(19) World Intellectual Property Organization International Bureau





(43) International Publication Date 28 July 2005 (28.07.2005)

PCT

(10) International Publication Number WO 2005/067632 A3

(51) International Patent Classification: A61K 9/127 (2006.01) C12N 15/88 (2006.01) C07H 21/04 (2006.01)

(21) International Application Number:

PCT/US2005/000418

(22) International Filing Date: 7 January 2005 (07.01.2005)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data: 60/535,042 60/556,843

60/557,232

7 January 2004 (07.01.2004) US 27 March 2004 (27.03.2004) US 29 March 2004 (29.03.2004) US

(71) Applicant (for all designated States except US): NEOPHARM, INC. [US/US]; 150 Field Drive, Suite 195, Lake Forest, Illinois 60045 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): AHMAD, Moghis U. [US/US]; 3050 North Forest Hills Ct., Wadsworth, Illinois 60083 (US). SHEIKH, Saifuddin [US/US]; 1059 South West Avenue, Waukegan, Illinois 60085 (US). ALI, Shoukath [IN/US]; 29681 North Waukegan Road, #204, Lake Bluff, Illinois 60044 (US). CHIEN, Pei-Yu [US/US]; 760 Pleasant Avenue, Highland Park, Illinois 60035 (US). JAMIL, Haris [US/US]; 1216 Trinity Place, Libertyville, Illinois 60048 (US). ZHANG, Jia-Ai [US/US]; 1251 Maidstone, Vernon Hills, Illinois 60061 (US). UGWU, Sydney [NG/US]; 4005 Stoney Island, Gurnee, Illinois 60031 (US). ZHANG, Zhi-Yi [CN/US]; 7986 Dada Drive, Gurnee, Illinois 60031 (US). WANG, Jinkang [US/US]; 39 Inverness Drive, San Francisco, California 94132 (US).

BHAMIDIPATI, Shastri [US/US]; 2380 Chambound Drive, Buffalo Grove, Illinois 60089 (US). AHMAD, Zafeer [US/US]; 2933 North Augusta Drive, Wadsworth, Illinois 60083 (US). AHMAD, Imran [US/US]; 4731 West Pebble Beach, Wadsworth, Illinois 60083 (US).

- (74) Agents: HEFNER, M. Daniel et al.; LEYDIG, VOIT & MAYER, LTD., 180 North Stetson Avenue, Two Prudential Plaza, Suite 4900, Chicago, Illinois 60601-6780 (US).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SM, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published

- with international search report
- with sequence listing part of description published separately in electronic form and available upon request from the International Bureau
- (88) Date of publication of the international search report: 2 April 2009

(54) Title: LIPID COMPOSITIONS AND USE THEREOF

(57) Abstract: The invention provides a composition suitable for use as a transfection agent, comprising a cationic cardiolipin analogue and, another lipid species. The composition of the present invention can facilitate transfection of a wide variety of polynucleotide species (e.g., oligodeoxyribonucleotides, plasmids, RNAi species, etc.). Moreover, the transfection agent of the present invention is effective in promoting transfection of primary cell cultures as well as transformed cells. Also, the inventive transfection agent is suitable for use both *in vitro* and *in vivo*. The inventive composition has additional uses as well, such as delivery of a variety of active agents, dermatological and cosmetic uses, and uses in agriculture. The invention further provides a method of introducing an active agent into a cell by contacting the cell with the inventive composition. The invention further provides a method of inhibiting the growth of neoplastic cells and a method of treating a patient suffering from a neoplastic disease by employing the inventive composition, wherein an active agent is an antineoplastic agent. The invention further provides a method for validating a genetic target, comprising (a) administering to a cell a composition comprising a cationic liposome and a siRNA, whereby the siRNA enters the cell inhibits the expression of a gene within the cell and (b) assaying for the inhibition of the gene. The method also provides a fluorescent/ luminescent cationic cardiolipin analogue and compositions including such analogues. Using a cationic cardiolipin analogue, the invention provides a method of tracking the migration of a lipid substance within an animal.